CIRM & NHLBI Create Landmark Agreement on Curing Sickle Cell Disease

Posted: April 30, 2019

CIRM Board approves first program eligible for co-funding under the agreement

Oakland, CA – Sickle Cell disease (SCD) is a painful, life-threatening blood disorder that affects around 100,000 people, mostly African Americans, in the US. Even with optimal medical care, SCD shortens expected lifespan by decades. It is caused by a single genetic mutation that results in the production of "sickle" shaped red blood cells. Under a variety of environmental conditions, stress or viral illness, these abnormal red blood cells cause severe anemia and blockage of blood vessels leading to painful crisis episodes, recurrent hospitalization, multi-organ damage and mini-strokes.

On April 29th the governing Board of the California Institute for Regenerative Medicine (CIRM) approved $4.49 million to Dr. Mark Walters at UCSF Benioff Children's Hospital in Oakland to pursue a gene therapy cure for this devastating disease. The gene therapy approach uses CRISPR-Cas9 technology to correct the genetic mutation that leads to sickle cell disease. This program will be eligible for co-funding under the landmark agreement between CIRM and the National Heart, Lung and Blood Institute (NHLBI) of the NIH.

This CIRM-NHLBI agreement was finalized this month to co-fund cell and gene therapy programs under the NIH "Cure Sickle Cell" initiative. The goal is to markedly accelerate the development of cell and gene therapies for SCD. It will deploy CIRM's resources and expertise that has led to a portfolio of over 50 clinical trials in stem cell and regenerative medicine.

"CIRM currently has 23 clinical stage programs in cell and gene therapy. Given the advancements in these approaches for a variety of unmet medical needs, we are excited about the prospect of leveraging this to NIH-NHLBI's Cure Sickle Cell Initiative," says Maria T. Millan, M.D., the President and CEO of CIRM. "We are pleased the NHLBI sees value in CIRM's acceleration and funding program and look forward to the partnership to accelerate cures for sickle cell disease."

"There is a real need for a new approach to treating SCD and making life easier for people with SCD and their families," says Adrienne Shapiro, the mother of a daughter with SCD and the co-founder of Axis Advocacy, a sickle cell advocacy and education organization. "Finding a cure for Sickle Cell would mean that people like my daughter would no longer have to live their life in short spurts, constantly having their hopes and dreams derailed by ER visits and hospital stays. It would mean they get a chance to live a long life, a healthy life, a normal life."

CIRM is currently funding two other clinical trials for SCD using different approaches. One of these trials is being conducted at City of Hope and the other trial is being conducted at UCLA.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov

Source URL: https://www.cirm.ca.gov/about-cirm/newsroom/press-releases/04302019/cirm-nhlbi-create-landmark-agreement-curing-sickle-cell